



ENDOCYTE

The future of precision medicine

PSMA-617 License Transaction

October 2, 2017

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Transformative transaction accelerates path to commercialization



1

Significant patient need addressing \$1Bn+ market opportunity

2

First in class targeted agent with high response rates

3

Strong strategic fit

Near-Term Focus

- Radioligand Therapy (RLT)
 - ^{177}Lu -PSMA-617 Phase 3 study in metastatic castration-resistant prostate cancer (mCRPC)
- CAR-T (Adaptor Controlled)
 - Phase 1 proof-of-concept (PoC) in osteosarcoma

Long-Term Opportunities

- Radioligand Therapy (RLT)
 - ^{177}Lu -PSMA-617 New Indication(s) in Earlier Lines of mCRPC Therapy
 - PSMA-617 – New Targets and/or Isotopes for New Indications
- CAR-T (Adaptor Controlled)
 - New CAR-T adaptor molecules (e.g. CAIX)
 - Additional solid tumors

A Transformative Transaction

Transaction Details



- Endocyte has licensed exclusive worldwide rights to develop and commercialize PSMA-617 from ABX GmbH (ABX). Consideration includes:
 - \$12M upfront payment to ABX
 - 2 million shares of Endocyte common stock issued to ABX at closing
 - Warrant to purchase up to 4 million shares of Endocyte common stock issued to ABX at closing
 - \$160M in potential regulatory and commercial milestones
 - Tiered royalties to ABX starting in mid-teens
- Endocyte will be responsible for 100% of future development expenses
- Hart-Scott-Rodino regulatory pre-approval has been obtained and the transaction is closed
- Endocyte year-end 2017 cash balance revised to >\$90M

¹⁷⁷Lu-PSMA-617 Addresses Significant Unmet Need in the Prostate Cancer Market



Prostate Cancer Market Opportunity

- 300,000 men die annually from metastatic castration-resistant prostate cancer (mCRPC)
 - ~100,000 in seven major markets of which ~80,000 are PSMA positive; a \$5Bn market at prices near comparative therapy
- Despite advances in therapies, which often slow the disease, patients who progress are left with limited treatment options
- Once metastasized, prostate cancer is nearly always lethal

¹⁷⁷Lu-PSMA-617

- ¹⁷⁷Lu-PSMA-617 pairs a PSMA targeting ligand (PSMA-617) to a radioactive isotope (¹⁷⁷Lu)
- The targeting ligand binds to PSMA expressed on diseased cells and releases an energetic beta particle that results in lethal radiation killing the cancer cell
- ¹⁷⁷Lu-PSMA-617 was developed at DKFZ and University Hospital Heidelberg
- ~20 peer reviewed publications in the post-chemotherapy compassionate use setting, highlight ¹⁷⁷Lu-PSMA-617's consistent meaningful PSA response (>50% decline from baseline) in 40% to 60% of patients, and a RECIST response rate in soft tissue disease of between 40% and 50%.

On September 29, 2017, ABX GmbH and Endocyte, Inc entered into an exclusive global licensing agreement whereby Endocyte will develop and commercialize PSMA-617

^{177}Lu -PSMA-617: Retrospective Data Analysis

German Multi-Center Study in 145 mCRPC patients

German Multicenter Study Investigating ^{177}Lu -PSMA-617 Radioligand Therapy in Advanced Prostate Cancer Patients

Kambiz Rahbar, Hojjat Ahmadzadehfar, Clemens Kratochwil, Uwe Haberkorn, Michael Schäfers, Markus Essler, Richard P. Baum, Harshad R. Kulkarni, Matthias Schmidt, Alexander Drzezga, Peter Bartenstein, Andreas Pfestroff, Markus Luster, Ulf Lützen, Marlies Marx, Vikas Prasad, Winfried Brenner, Alexander Heinzel, Felix M. Mottaghy, Juri Ruf, Philipp Tobias Meyer, Martin Heuschkel, Maria Eveslage, Martin Bögemann, Wolfgang Peter Fendler and Bernd Joachim Krause

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- Study included all patients with PSMA positive disease without regard to potential presence of PSMA negative disease
- Serial PSA levels for analyzing responses were available in 99 of 145 patients
- After the first therapy cycle, a PSA decline of $\geq 50\%$ occurred in 40 of 99 patients (40%)
- After the second therapy cycle, a PSA decline of $\geq 50\%$ occurred in 35 of 61 patients (57%)

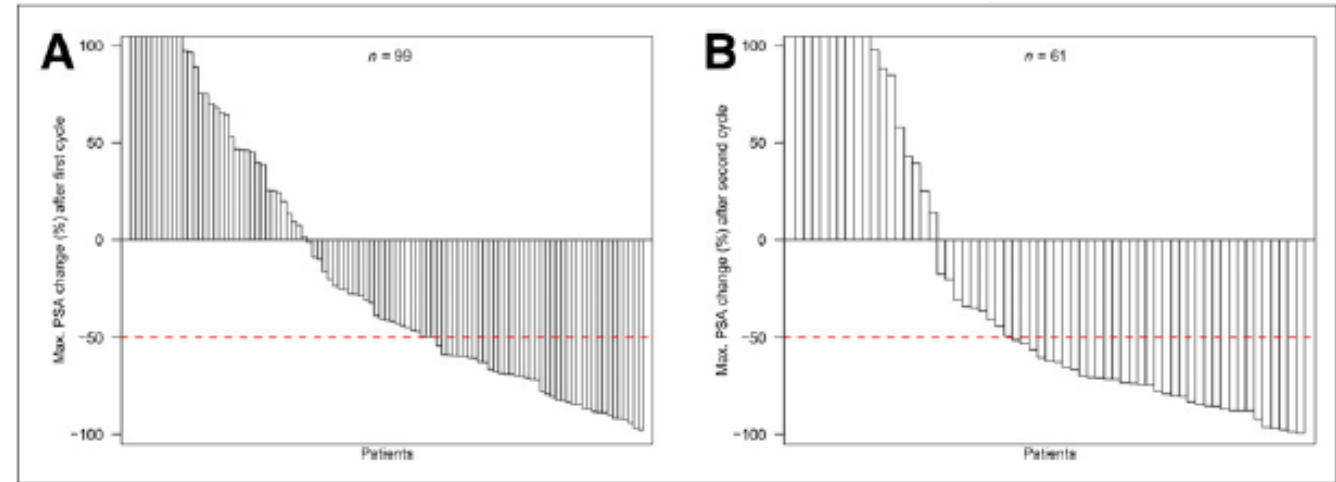


FIGURE 3. Waterfall plots of maximum PSA change (%) after first cycle (A) and after second cycle (B). PSA increase $> 100\%$ was cropped due to simplification.

¹⁷⁷Lu-PSMA-617: retrospective safety data analysis in mCRPC patients (n = 145)

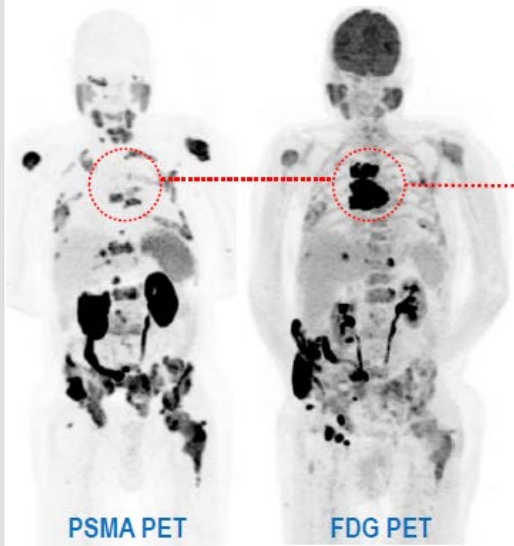
TABLE 3
Adverse Events After ¹⁷⁷Lu-PSMA-617 as Determined by Blood Tests (n = 121) or Physician Reports (n = 145)

Organ system	Category	Evaluated for N	All grades	Grade 3–4
Blood and lymphatic disorders	Leukopenia	121	48 (40%)	4 (3%)
	Anemia	145	50 (34%)	15 (10%)
	Thrombocytopenia	121	38 (31%)	5 (4%)
Gastrointestinal disorders	AST elevation	121	27 (19%)	0 (0%)
	ALT elevation	121	11 (8%)	0 (0%)
	Xerostomia	145	11 (8%)	0 (0%)
	Nausea	145	9 (6%)	0 (0%)
	Dysgeusia	145	6 (4%)	0 (0%)
	Ascites	145	2 (1%)	0 (0%)
	Biliary obstruction	145	0 (0%)	1 (1%)
General disorders	Fatigue	145	19 (13%)	1 (1%)
	Pain	145	5 (3%)	0 (0%)
	Ileus	145	1 (1%)	0 (0%)
Urinary disorders	Renal failure	121	14 (12%)	0 (0%)
	Urinary tract infection	145	1 (1%)	0 (0%)
Cardiovascular disorders	Edema	145	2 (1%)	0 (0%)
	Lung embolism	145	0 (0%)	3 (2%)
Respiratory, thoracic, and mediastinal disorders	Pleural effusion	145	1 (1%)	0 (0%)
	Dyspnea	145	1 (1%)	0 (0%)
Neurologic disorders	Vertigo	145	1 (1%)	0 (0%)
	Stroke	145	0 (0%)	2 (1%)
Musculoskeletal disorders	Bone fracture	145	0 (0%)	3 (2%)

^{177}Lu -PSMA-617: Prospective Clinical Data

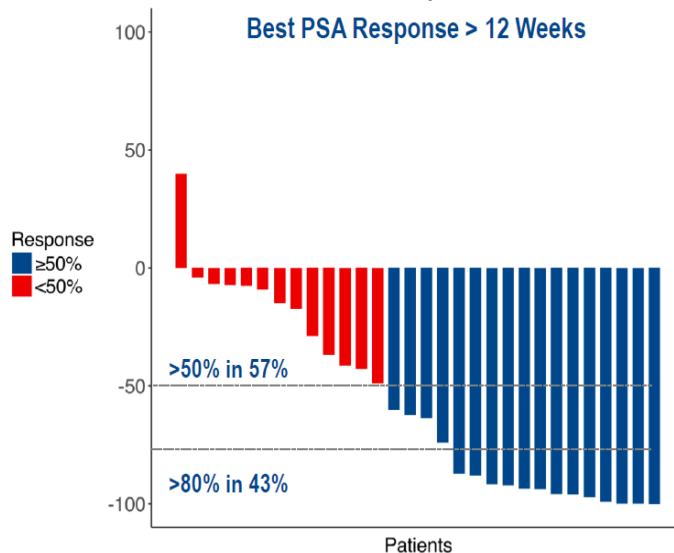
Results Presented at ESMO Garner Significant Attention from Investigators⁽¹⁾

Refined Patient Selection



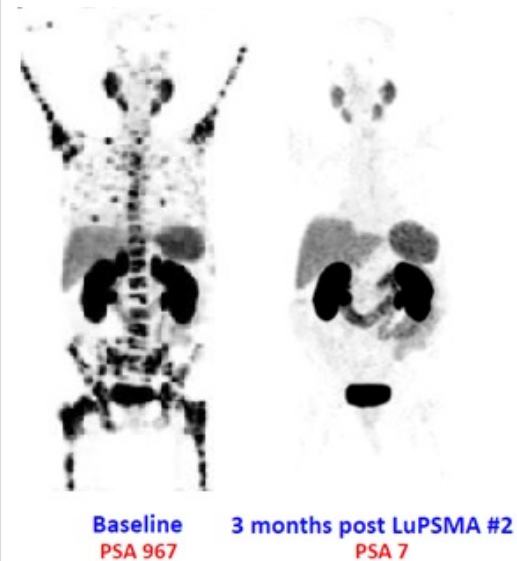
Patient excluded from trial. PSMA negative disease appears on FDG PET and not on PSMA PET.

Driving Response



71% RECIST response (n=17) among patients with soft tissue disease

Post Treatment Scan



PSMA positive disease not visibly detected from follow-up scan.

^{177}Lu -PSMA-617 prospective safety data analysis in mCRPC patients (n = 30)

Low incidence rates of grade 3/4 hematological and non-hematological toxicities attributable to ^{177}Lu -PSMA-617

Non haematological attributable to LuPSMA:

Toxicity	G1/2 (%)	G3/4 (%)
Dry mouth	63	0
Nausea*	50	0
Vomiting*	20	0
Fatigue	17	3
Dry eyes	7	0
Bone pain	7	3
Anorexia	7	0
Infusion related reactions	0	0
Renal toxicity	0	0

Haematotoxicity:

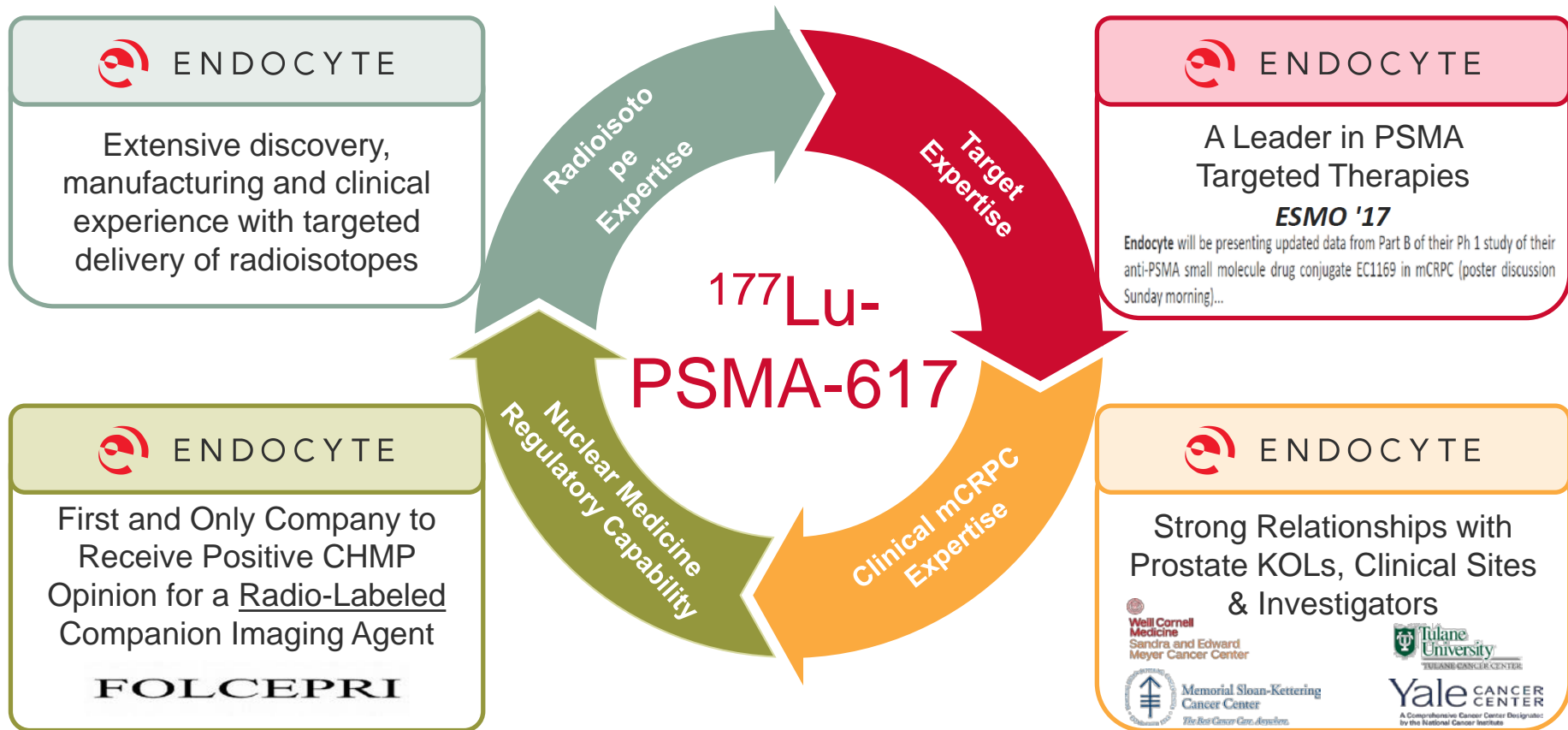
Toxicity	G1/2 (%) (baseline)	G1/2 (%) (any cause)	G3/4 (%)	G3/4 (%) (LuPSMA)
Haemoglobin	80	73	23	7
Neutrophils**	0	40	10	7
Platelets	17	43	27	13

* transient and self-limiting within first 24 hours

** no episodes of febrile neutropenia

CTCAE version 4: adverse events that occurred within 12 weeks after last injection of LuPSMA, or more than 12 weeks if determined to be related to LuPSMA

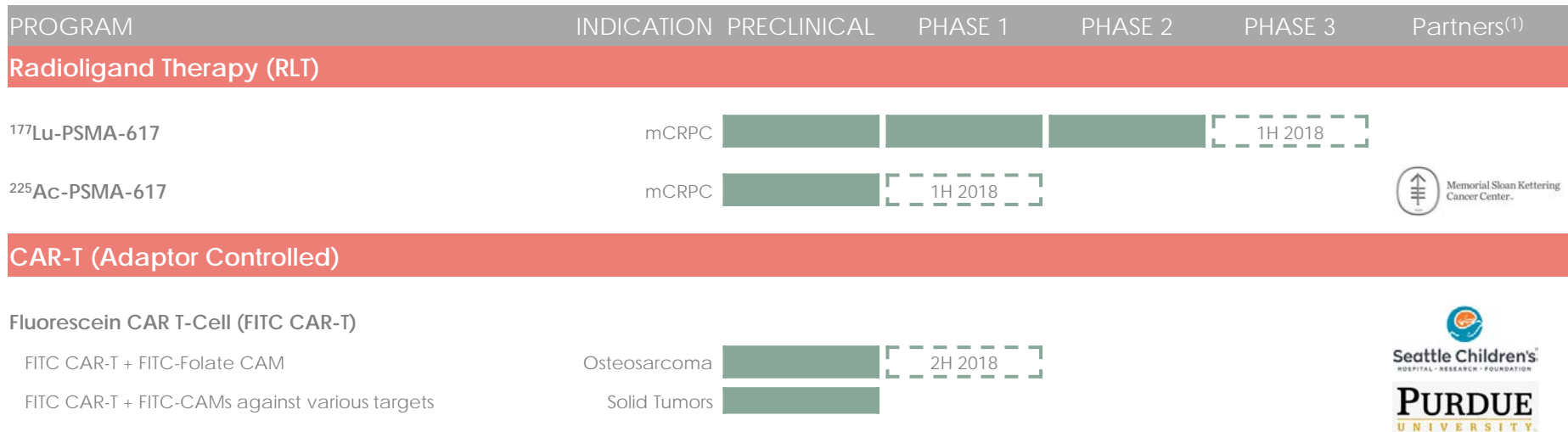
Development of ^{177}Lu -PSMA-617 will leverage Endocyte's experience and capabilities



¹⁷⁷Lu-PSMA-617 has the potential to achieve many differentiating 'firsts':

-  First targeted radioligand therapy in prostate cancer
-  First therapeutic targeting PSMA of any kind
-  First ¹⁷⁷Lu therapeutic agent in prostate cancer
-  First radiotherapy targeting both bone & soft tissue disease in prostate cancer

Focused development of targeted therapeutics for cancer treatment



⁽¹⁾ The work of partner programs is being undertaken by the partner with modest funding from Endocyte. Endocyte's near-term focus is on ¹⁷⁷Lu-PSMA-617

Transaction highlights

Positions Endocyte as the Leader in Prostate Cancer Radioligand Therapy (RLT)

- Innovative therapeutic approach to treatment of mCRPC
- Meaningful results to date garner attention from investigators and patients
- Highly complementary to Endocyte's existing capabilities

\$1Bn+ Peak Revenue Opportunity

- Large post-chemotherapy mCRPC market with limited treatments available
- ~80,000 PSMA positive patients die annually in 7 major markets; \$5Bn market
- Well established referral base and understanding of radiotherapy treatment modalities between urologists, medical oncologists and radiologists

Focused Resources

- Endocyte has the focus and ability to fund both priority pipeline assets
- Phase 3 registration trial in ¹⁷⁷Lu-PSMA-617
- Targeted adaptor controlled CAR-T proof of concept in solid tumors

Significant Long-Term Upside Potential

- ¹⁷⁷Lu-PSMA-617 earlier lines of therapy, or different isotope
- ²²⁵Actinium alpha-emitter isotope w/ MSKCC¹
- CAR-T expansion with adaptors for other solid tumor targets (e.g. NK1R)



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